

Abstract

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Systematic review of pharmacological treatments in fragile X syndrome.

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BACKGROUND: Fragile X syndrome (FXS) is considered the most common cause of inherited mental retardation. Affected people have mental impairment that can include Attention Deficit and/or Hyperactivity Disorder (ADHD), autism disorder, and speech and behavioural disorders. Several pharmacological interventions have been proposed to treat those impairments.

METHODS: Systematic review of the literature and summary of the evidence from clinical controlled trials that compared at least one pharmacological treatment with placebo or other treatment in individuals with diagnosis of FXS syndrome and assessed the efficacy and/or safety of the treatments. Studies were identified by a search of PubMed, EMBASE and the Cochrane Databases using the terms fragile X and treatment. Risk of bias of the studies was assessed by using the Cochrane Collaboration criteria.

RESULTS: The search identified 276 potential articles and 14 studies satisfied inclusion criteria. Of these, 10 studies on folic acid (9 with crossover design, only 1 of them with good methodological quality and low risk of bias) did not find in general significant improvements. A small sample size trial assessed dextroamphetamine and methylphenidate in patients with an additional diagnosis of ADHD and found some improvements in those taking methylphenidate, but the length of follow-up was too short. **Two studies on L-acetylcarnitine, showed positive effects and no side effects in patients with an additional diagnosis of ADHD.** Finally, one study on patients with an additional diagnosis of autism assessed ampakine compound CX516 and found no significant differences between treatment and placebo. Regarding safety, none of the studies that assessed that area found relevant side effects, but the number of patients included was too small to detect side effects with low incidence.

CONCLUSION: Currently there is no robust evidence to support recommendations on pharmacological treatments in patients with FXS in general or in those with an additional diagnosis of ADHD or autism.

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